geneic transplant due to older age, comorbidities or lack of HLA-identical donor. Median age at transplant was 52 years (range 37–68), median interval between first and second autograft was 26 months (range 3–73). Eleven patients had persistent or progressive disease at transplant. Twenty patients received high-dose melphalan alone or in combination, while 2 received a combination of thiotepa, busulfan and cyclophosphamide. Patients had received an average of 6 prior chemotherapy regimens. Cytogenetic studies were available in 18/22 patients at the time of transplant, 12 were normal and 6 abnormal. **Results:** 14 of the 21 evaluable patients (70%) achieved a response (1 CR, 13 PR). After a median follow-up of 15 months (2–82), 1 year progression-free survival (PFS) was 40% and 1-year overall survival (OS) was 78%. 100-day TRM was 0%. Median PFS was 10 months, and median OS has not been reached. On univariate analysis, abnormal cytogenetics at transplant was a predictor of shorter overall survival ($P = .01$). **Conclusions:** In patients with progressive disease after an autologous transplant, salvage autologous transplants may achieve responses in 70% of patients with durable remissions in a small subset. Normal cytogenetics at second transplant predicts a longer survival.

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### AUTOLOGOUS PERIPHERAL BLOOD STEM CELL TRANSPLANTATION IN MULTIPLE MYELOMA USING MELPHALAN: THE MEXICAN EXPERIENCE

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**Background:** Autologous peripheral blood stem cell transplantation is at the present the therapy of choice for the treatment of multiple myeloma (MM) patients younger than 70 years old. **Methods:** Between August 1993 and November 2004, fifty four patients with MM were autografted; median age was 49 years (range 20–69). Patients were given a median of $4.3 \times 10^6$/Kg CD34 (+) viable MNC after conditioning with high-dose melphalan regimens (oral or I.V. in 47 and 7 patients, respectively). **Results:** Median time to achieve $> 0.5 \times 10^9$/L granulocytes was 12 days, whereas median time to achieve $>20 \times 10^9$/L platelets was 15 days. Thirty seven patients are alive 15 to 157 months after the autograft (median 86 months). The 7-year disease-free and overall post-transplant survival is 24% and 60%, respectively. The transplant-related mortality was 13%. Seven patients died as a result of the toxicity of the conditioning regimen, whereas death in the remaining 10 cases was related to post-transplant relapse of the malignancy. Four good-prognostic factors were identified: interval between diagnosis and transplant less than 24 months, number of prior chemotherapy regimens <2, remission status (complete or partial), and pretransplant β2microglobulin less than 3 mg/dL. **Conclusions:** Autologous peripheral blood stem cell transplantation using oral melphalan was a good choice of treatment for Mexican multiple myeloma patients. Transplant-related mortality was higher in comparison with other studies.

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### DRAMATIC IMPROVEMENT OF POEMS SYNDROME BY STEM CELL TRANSPLANTATION PARALLELS DECREASE IN VEGF AND BFGF LEVEL

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**Purpose:** POEMS syndrome is a rare disease characterized by polyneuropathy, organomegaly, endocrinopathy, M-protein, and skin changes. We treated a severely ill woman with a 4-year history of polyneuropathy showing all signs of a POEMS syndrome. Response to chemotherapy including high-dose melphalan treatment and autologous stem cell transplantation was monitored and vascular endothelial growth factor (VEGF) as well as basic fibroblast growth factor (bFGF) levels were measured. **Methods:** Blood investigation was done for serum electrophoresis analysis and analysis of VEGF, bFGF, and IL-6 by ELISA. Bone marrow biopsy specimen was investigated immunohistochemically for IgA, IgG, kappa, lambda, CD20, CD56, cyclin D1, and VEGF. **Results:** Immunohistochemical investigation of the bone marrow biopsy showed an infiltration of IgA and lambda positive plasma cells (10%). Only few plasma cells expressed kappa. The tumor cell were negative for CD20, CD56, and cyclin D1, but positive for VEGF in line with the high VEGF levels in the blood. Blood investigation revealed a discrete monoclonal gammopathy of IgA lambda type. Initially, high level of VEGF (1468.7 pg/ml) and bFGF (112.9 pg/ml) were detected. However, treatment with high-dose melphalan and tandem autologous stem cell transplantation proved extremely helpful in disease control. Already after the first transplant the patient started again walking and lost her pulmonary hypertension. In parallel VEGF and bFGF levels decreased and the performance status of the patient improved dramatically. **Conclusions:** VEGF and bFGF measurement is a useful tool for monitoring disease activity in POEMS syndrome. Moreover, although stem cell transplantation is of utmost importance even in patients with severely reduced performance status, these pathophysiologic findings may provide a rational for additional treatment approaches with anti-angiogenetic substances.

# PEDIATRIC DISORDERS

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### BONE MARROW TRANSPLANTATION FROM MATCHED UNRELATED DONORS FOR PATIENTS WITH SEVERE COMBINED IMMUNE DEFICIENCY

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**Introduction:** Bone Marrow Transplant (BMT) from related HLA identical donor (RID) is the treatment of choice for patients with severe combined immuno-deficiency (SCID). In the absence of RID, bone marrow from HLA haplo-identical (half) related donors (HID) have often been used. However, HID is frequently associated with reduced survival and failure of long-term immune reconstitution. HLA matched unrelated donors (MUD) represent another promising alternative for patients with SCID. **Methods:** We studied hematopoietic engraftment, occurrence of graft versus host disease, infections and other complication, and survival in infants diagnosed with SCID who received MUD BMT between 1990 and 2004 in a Canadian pediatric referral center specializing in such procedures. Detailed evaluations of immune reconstitution were performed in children that survived more than 2 years after transplant. **Results:** During the 14 years of this study, 22 infants underwent MUD BMT in our center. Molecular diagnosis was available in 64% of them. All infants received myelo-ablative conditioning pre-transplant. Despite prophylaxis with cyclosporine and methylprednisolone, acute graft versus host disease occurred in 15 of the 22 patients, and it was the most common cause of death. All patients developed complete donor lymphocyte engraftment. None of the 15 patients who are two or more years after transplant requires intravenous immunoglobulin replacement therapy. After discontinuing immune suppression all patients have normal T cell function and T cell repertoire, which is sustained for more than 14 years of follow up. Current survival is 73% and it is even better for patients with mutations in the IL-2 receptor pathway. Furthermore, survival of patients who presented with low B cells, previously estimated to have unfavorable outcome, is not significantly lower than in other forms of SCID in this study. **Conclusions:** MUD BMT leads to long term survival, engraftment, and immune function in SCID patients and should be the preferred treatment for clinically stable infants who do not have a related HLA-identical donor.